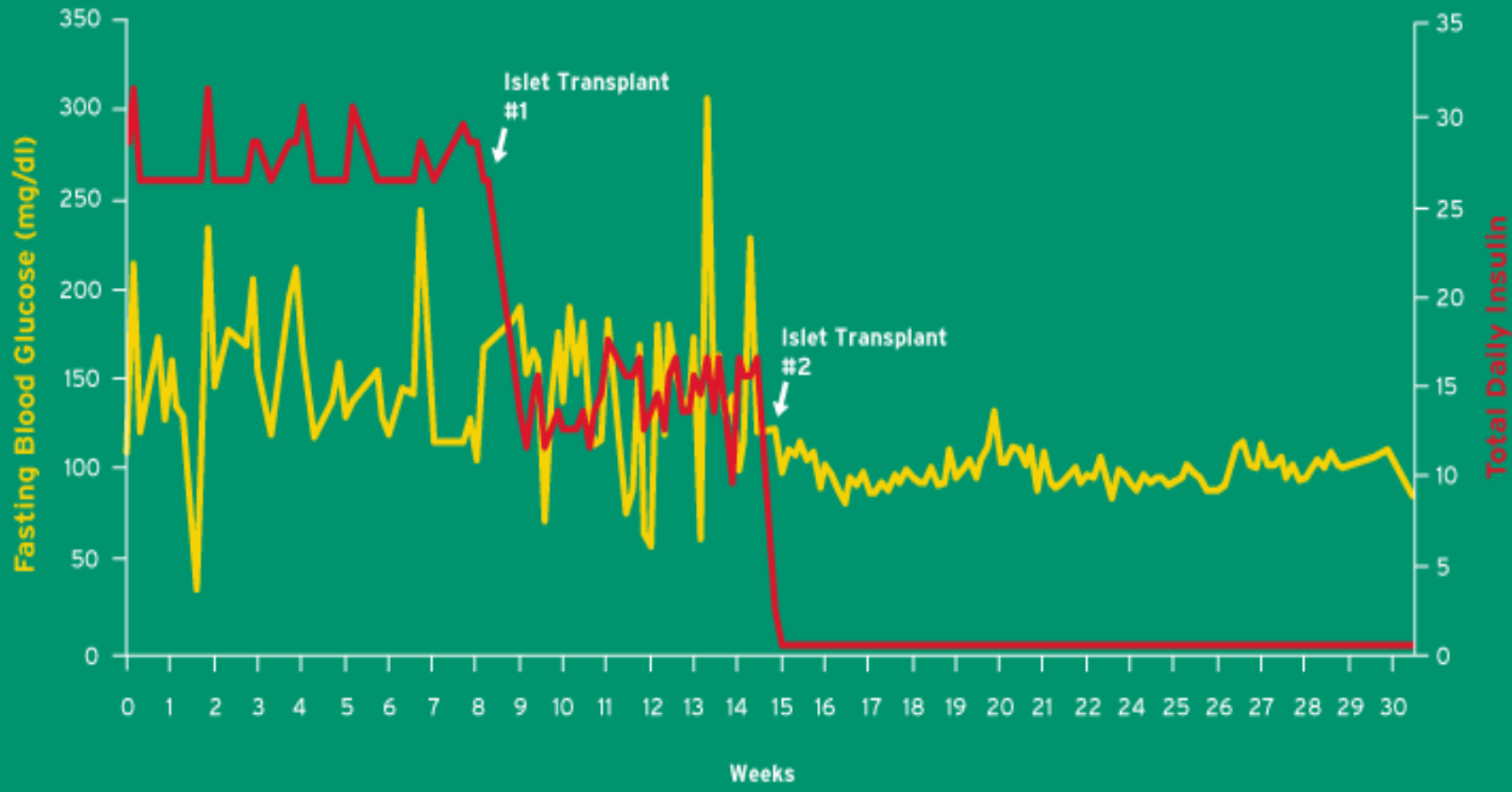
Delivering on the Promise of Stem Cell Research: What Will it Take?

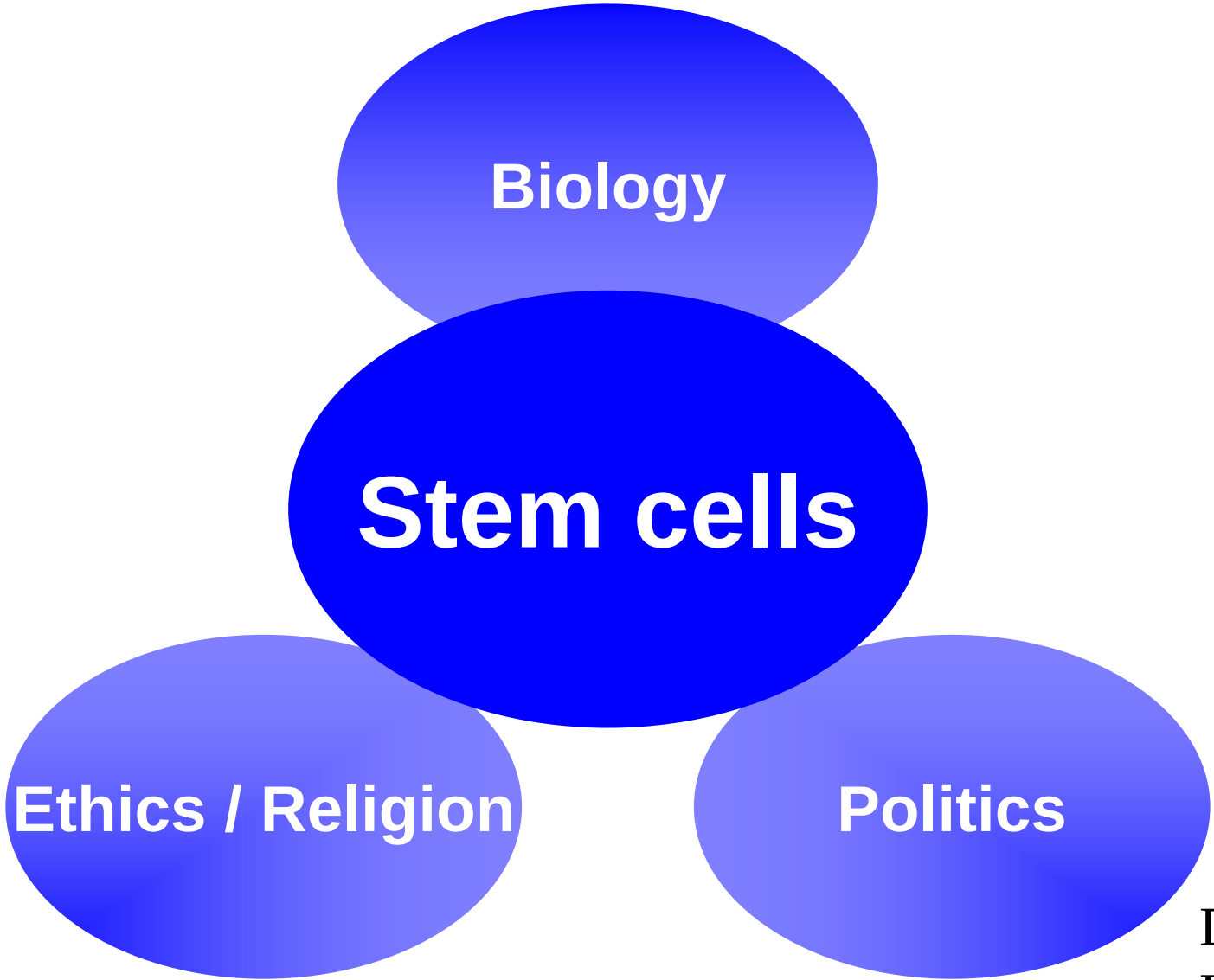
Allen M. Spiegel, M.D.
Dean
Albert Einstein College of Medicine

CIRM Conference
July 13, 2006

Fifty-seven Year Old Woman Diagnosed with T1DM in 1950 (Brittle)

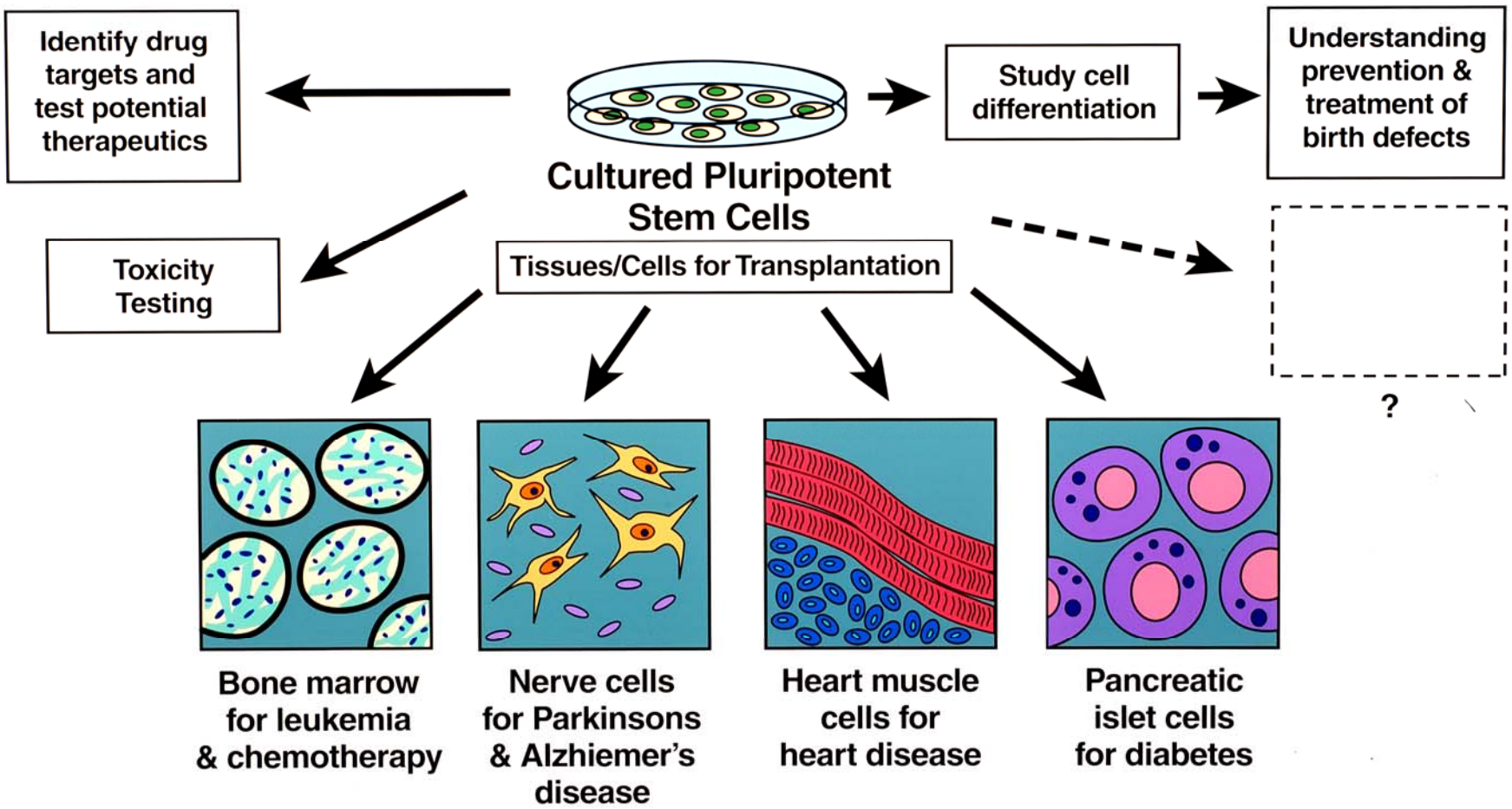


The Science and Public Policy of Stem Cells



D. Melton
Harvard

The Promise of Stem Cell Research



Stem Cell and Developmental Biology Writing Groups

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Jeffrey I. Gordon, M.D.
Sandra Puczynski, Ph.D.
Ming-Jer Tsai, Ph.D.

NIH Intramural Advisor:
Igor B. Dawid, Ph.D.

Challenges

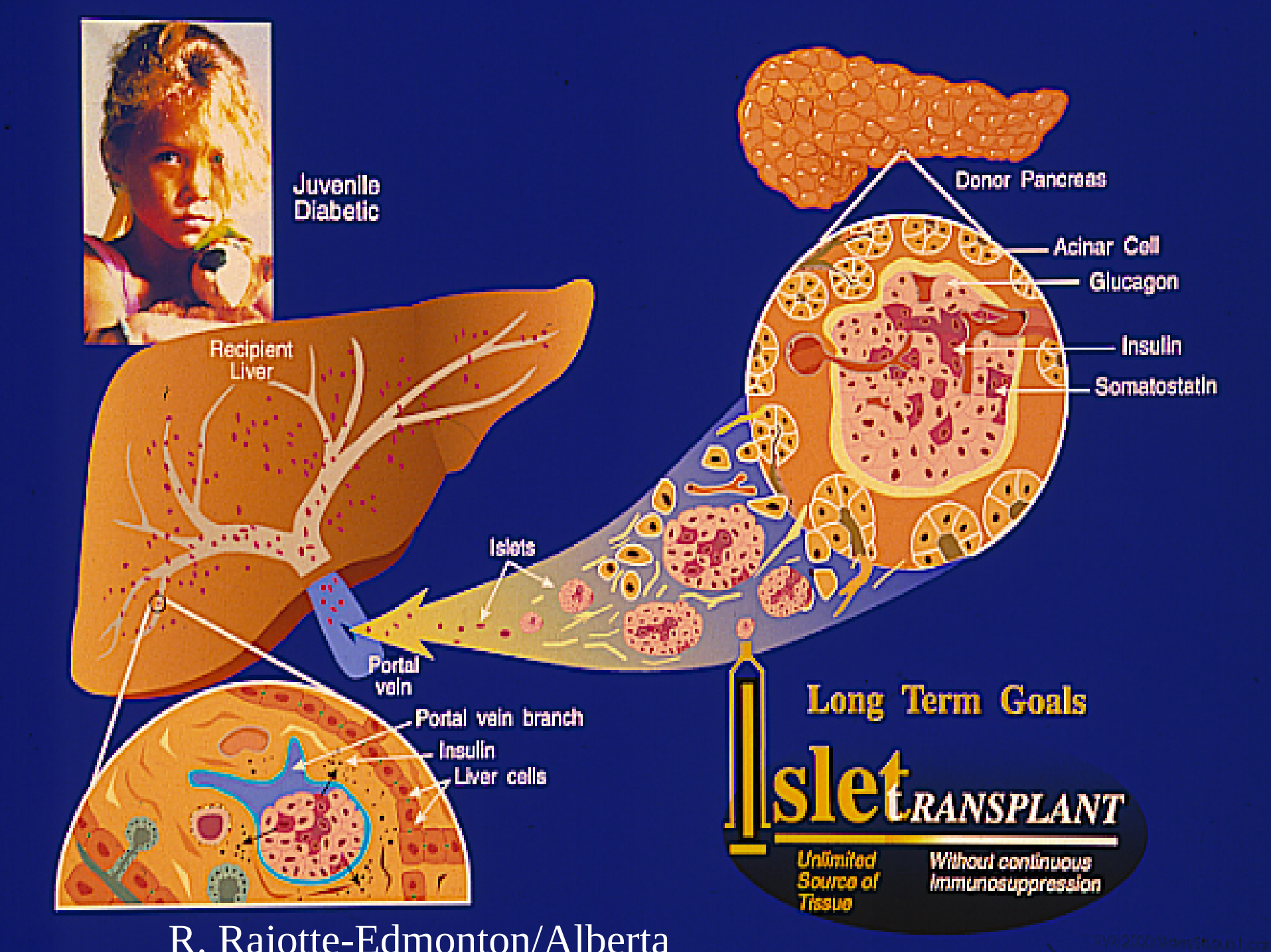
- New methods for recovering stem cells as well as other cell populations necessary to maintain ‘stemness’ *ex vivo*
- New ways of assaying stem cell functions *in vivo* and *ex vivo*
- Integrate genomics, proteomics, and bioinformatics to characterize molecular features of stem cells and their committed daughters
- New *in vivo* models for studying stem cell function

Proactive Role of NIDDK

- Development of enabling technologies and knowledge base
- Launch research initiatives that connect stakeholders from multiple disciplines across the country (scope/scale of science rapidly expanding in the early post-genomic era)
- Provide a means for making biological reagents from model organisms and humans available to the research community
- Assure adequate training of scientists and physician-scientists in areas supportive of stem cell research
- Adequate education of the public concerning the importance of this area of investigation.

Recommendation

“NIDDK should catalyze a nation-wide effort to characterize the molecular and cellular features of stem cells during and following development of the pancreas, liver, stomach and intestine, kidney and GU tract, bone and hematopoietic tissues”



R. Rajotte-Edmonton/Alberta

Obstacles and Opportunities on the Road to an Artificial Pancreas:

CLOSING THE LOOP

December 19, 2005
Lister Hill Auditorium
Bethesda, MD

JDRF

National Institutes of Health

National Institute of Diabetes & Digestive & Kidney Diseases

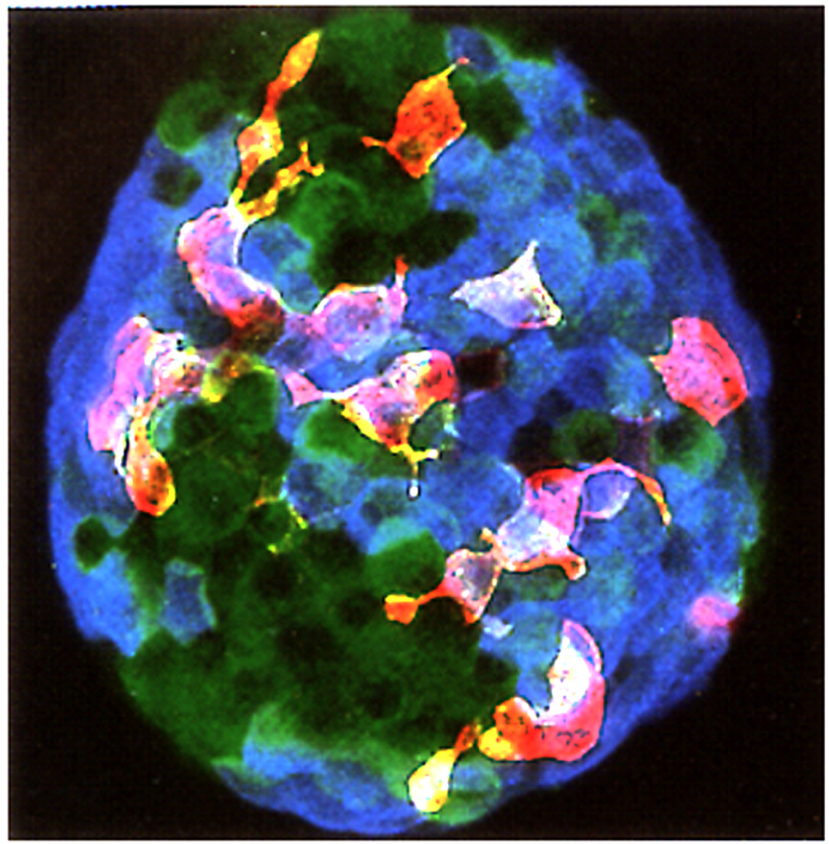
Pancreatic islet

insulin

glucagon

pancreatic polypeptide

somatostatin



D. Melton
Harvard

Beta Cell Biology Consortium

Mission:
To facilitate interdisciplinary approaches that will advance our understanding of pancreatic islet development and function.

Goal:
To develop a cell-based therapy for insulin delivery

www.betacell.org

NATIONAL INSTITUTES OF HEALTH

NIDDK

DEPARTMENT OF HEALTH & HUMAN SERVICES · USA

Beta Cell Biology Consortium

Your Account

Welcome

Guest

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Reagent Collections

- Antibodies (29)
- mESC Lines (0)
- Microarrays (4)
- Mouse Strains (13)

Data Resources

- EpConDB (25)
- TRAFEX (790)

Information

- About Us
- Investigators
- Research
- Publications
- Health

Click the image for details.

Welcome to the Beta Cell Biology Consortium

The mission of the Beta Cell Biology Consortium (BCBC) is to facilitate interdisciplinary approaches that will advance our understanding of pancreatic islet development and function with the long-term goal of developing a cell-based therapy for insulin delivery. (read more)

Meet the BCBC research investigators and explore our research, reagent collections and data resources, or browse our site map.

News & Events

Oct. 18, 2006 - Programming Pancreatic Beta Cells Workshop

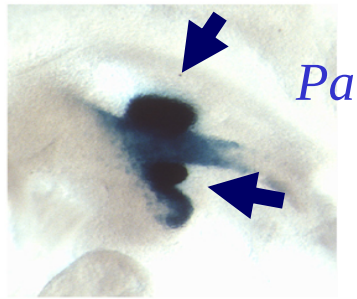
Sep. 14, 2006 - EASD 42nd Annual Meeting & Islet Study Group Symposium

Apr. 28, 2006 - Antibodies GN-ID4, GS-9A8 and Nkx6.1 Now Available to the Public

Jan. 04, 2006 - Mouse PromoterChip BCBC-5B Available

Nov. 02, 2005 - Resource Centers for Human Pancreatic Islets

Beta Cell Biology Consortium

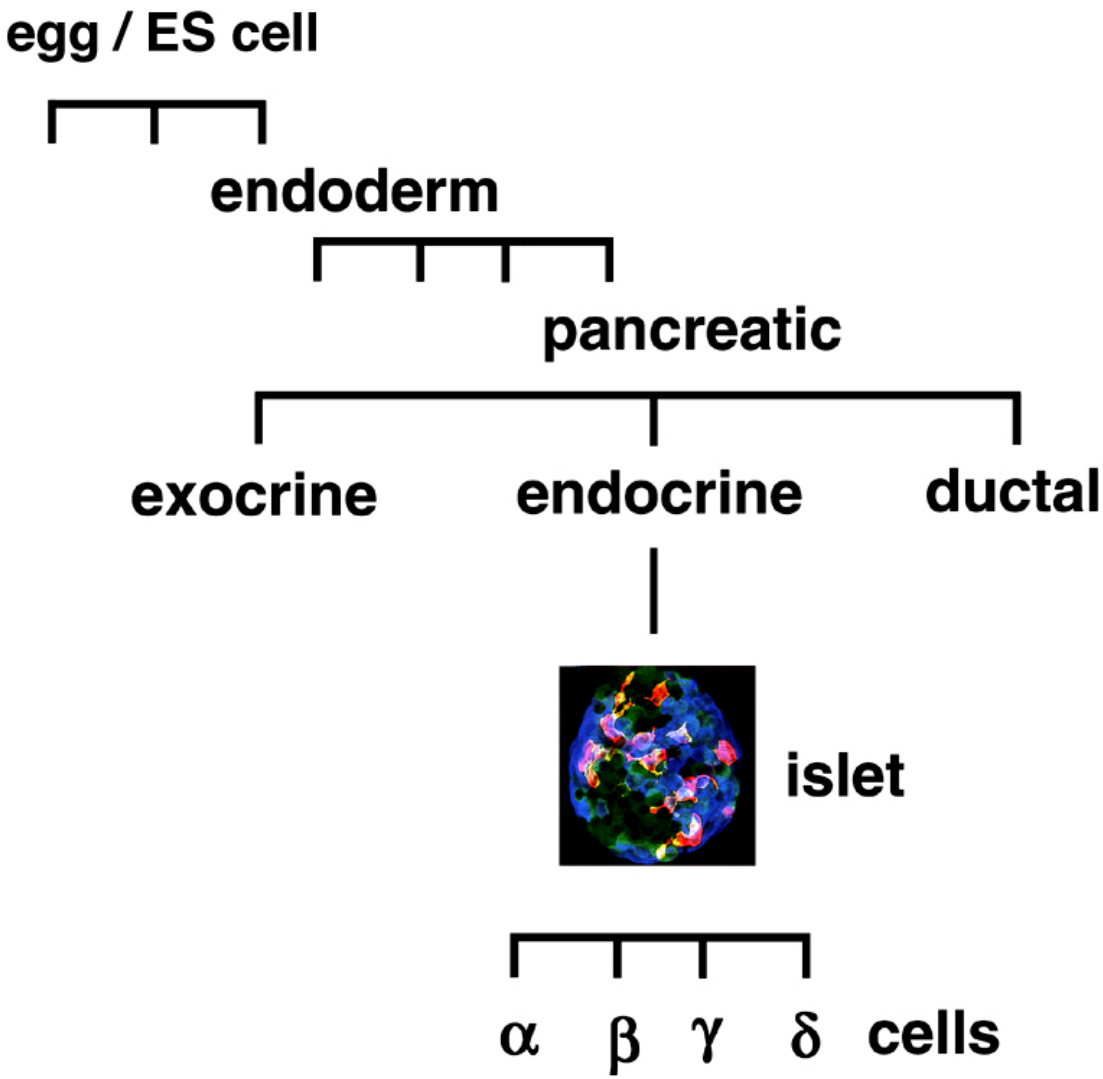


Pancreatic buds

Current Research

- Determine the temporal expression and function of genes during pancreatic islet development
- Develop tools to identify and prospectively isolate pancreatic stem /progenitor cells
- Identify factors that can drive stem cell differentiation toward a pancreatic progenitor lineage

Pancreatic islet production



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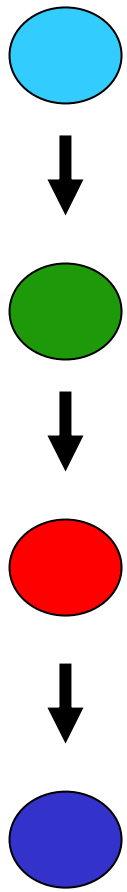
Converting ES cells into β cells

transfect candidate genes
and/or
add external signals



ES cell

Pdx1+

Ngn3+

Insulin+

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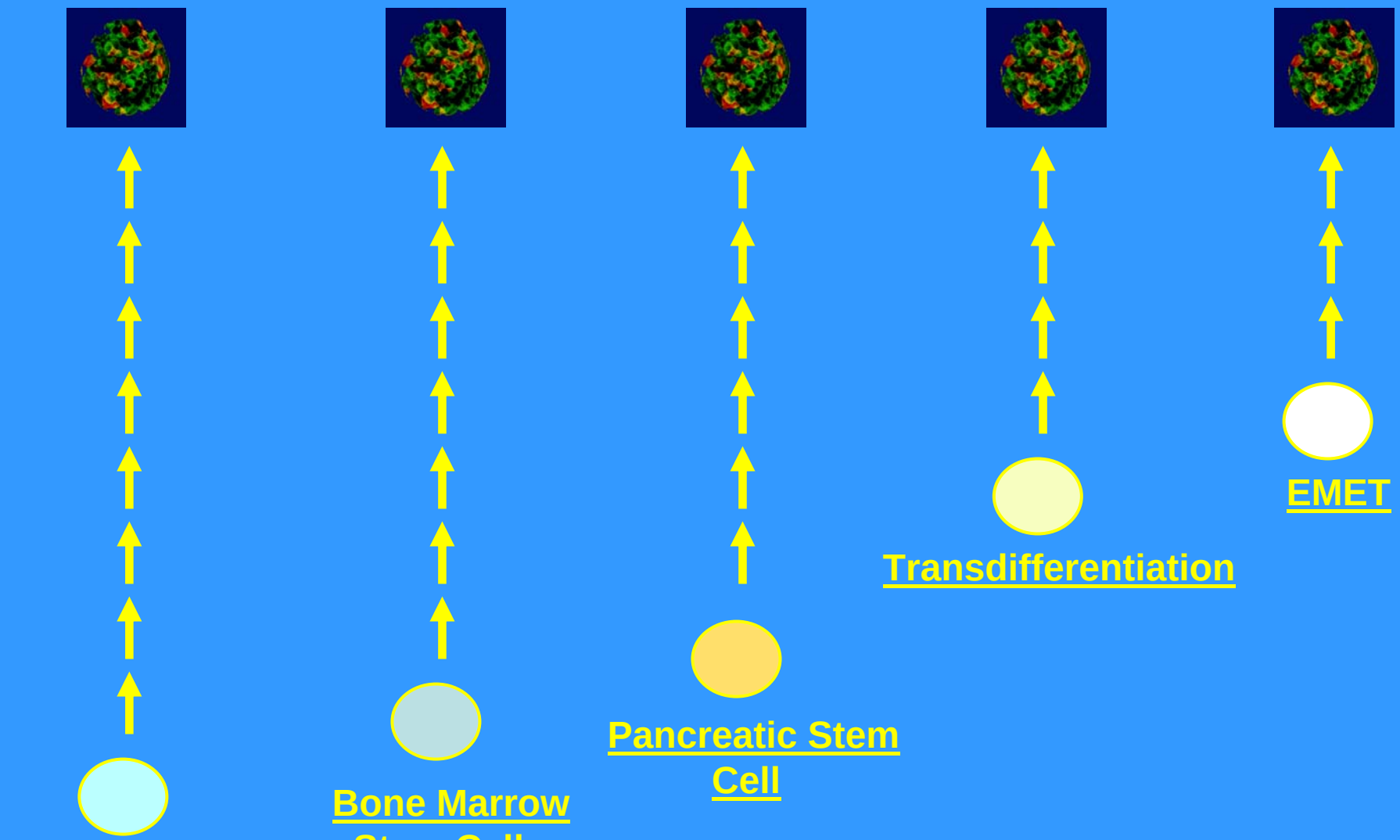


M. Gershengorn
NIDDK

Can pancreatic islet function recover in patients with long standing T1DM?

Data suggesting the possibility:

Functional- Persistent insulin production years after T1DM onset

Anatomical- Autopsy series showing pancreatic beta cells years after T1DM

Immunological- Immune cells "armed" for beta cells found in the lymph nodes that "drain" the pancreas years after T1DM onset

Technological advances allowing us to test the hypothesis:

Functional- Now possible to "tightly" control blood sugar

Immunological- New, safer, and more specific immune interventions

Other technical advances under development:

Anatomical- Techniques for measuring beta cell number in humans- *Dr. Liu*

"Supply side"- Agents purported to stimulate new beta cell growth in rodents

Intervention trial testing islet recovery hypothesis

Best modern insulin-based therapy

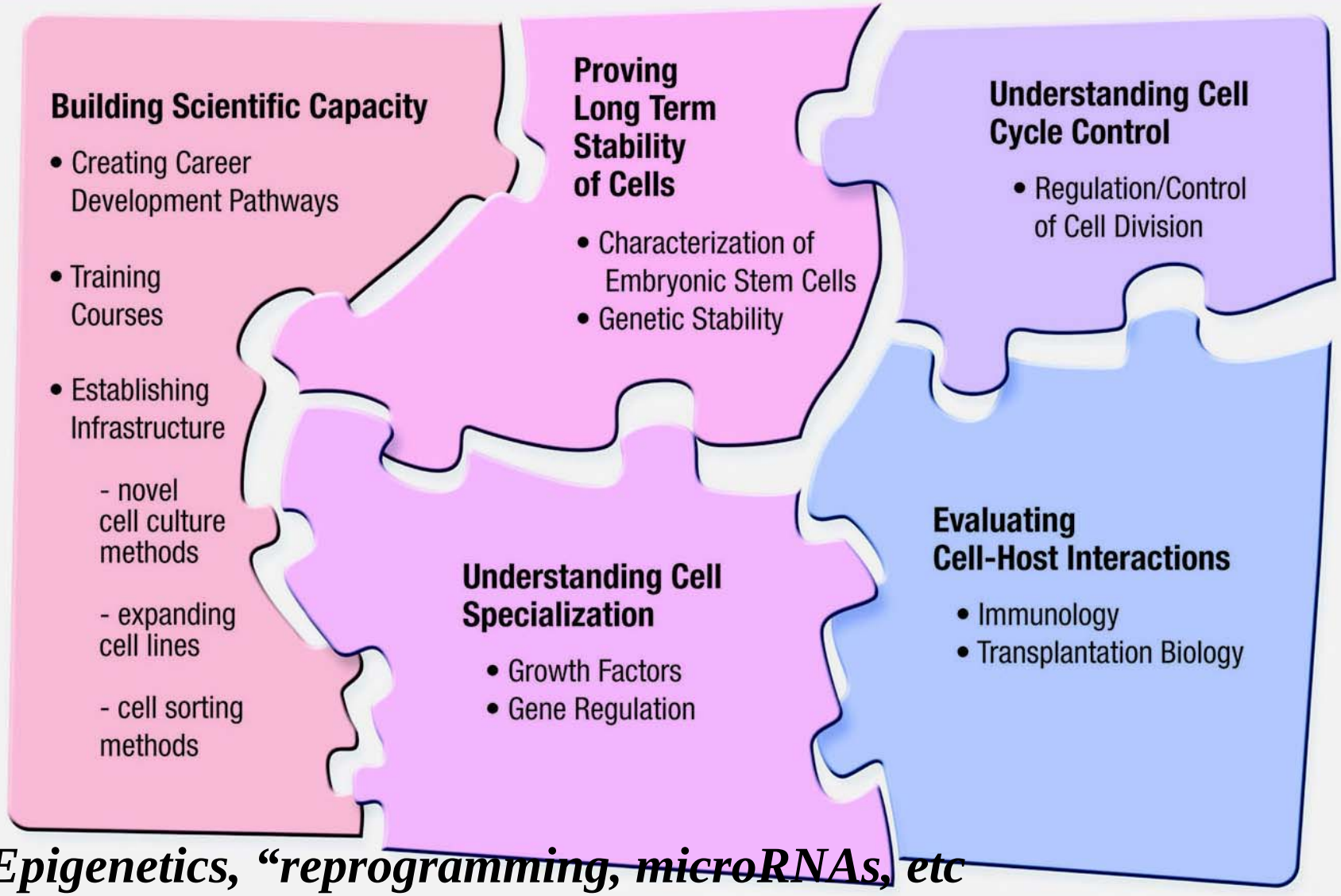
Agents to weaken immune system
±
Agents promoting β cell recovery

Patient with long-standing T1DM

Insulin production improved?

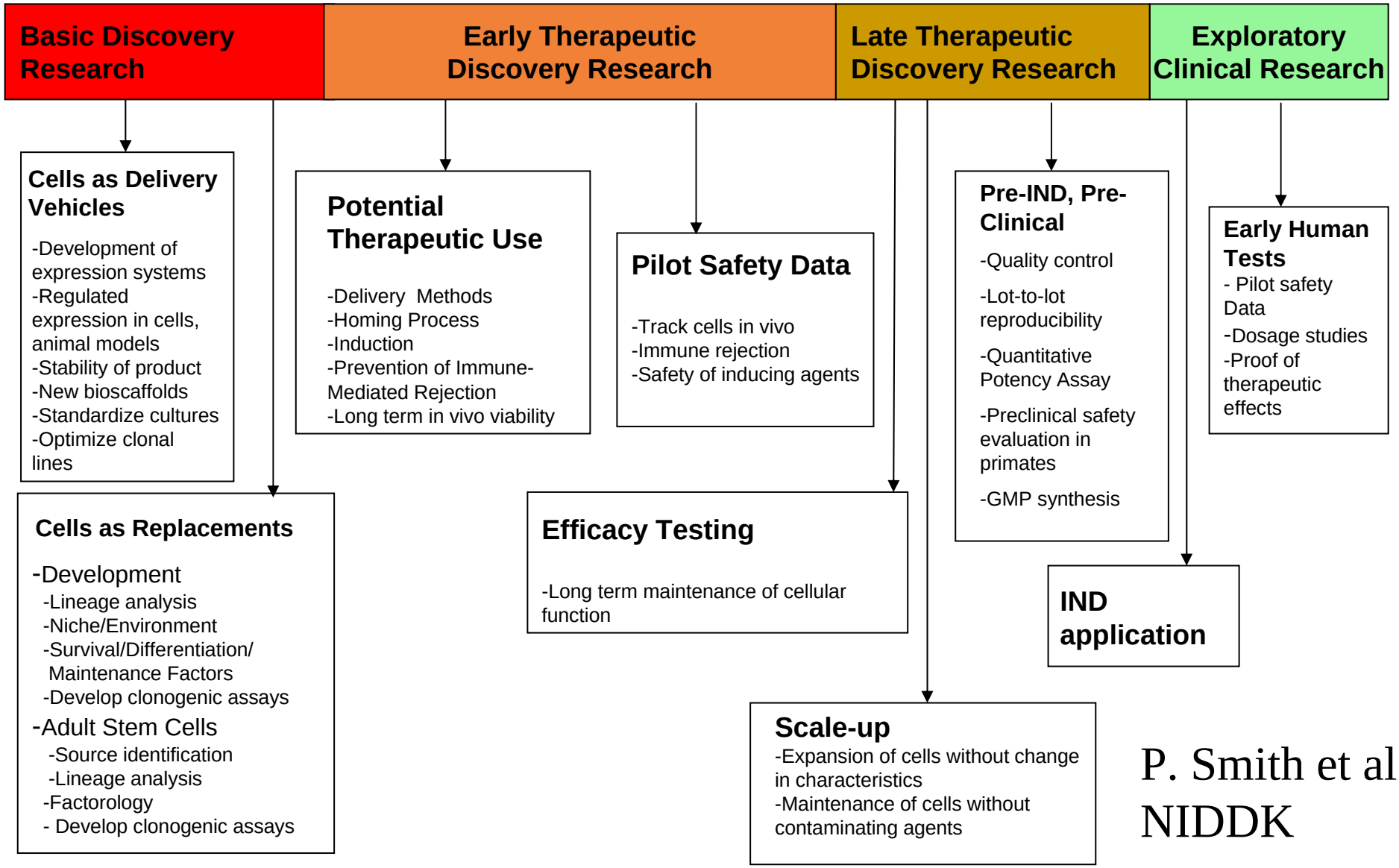
The Scientific Challenges of Human Stem Cells

Basic Research Phase →



Epigenetics, "reprogramming, microRNAs, etc

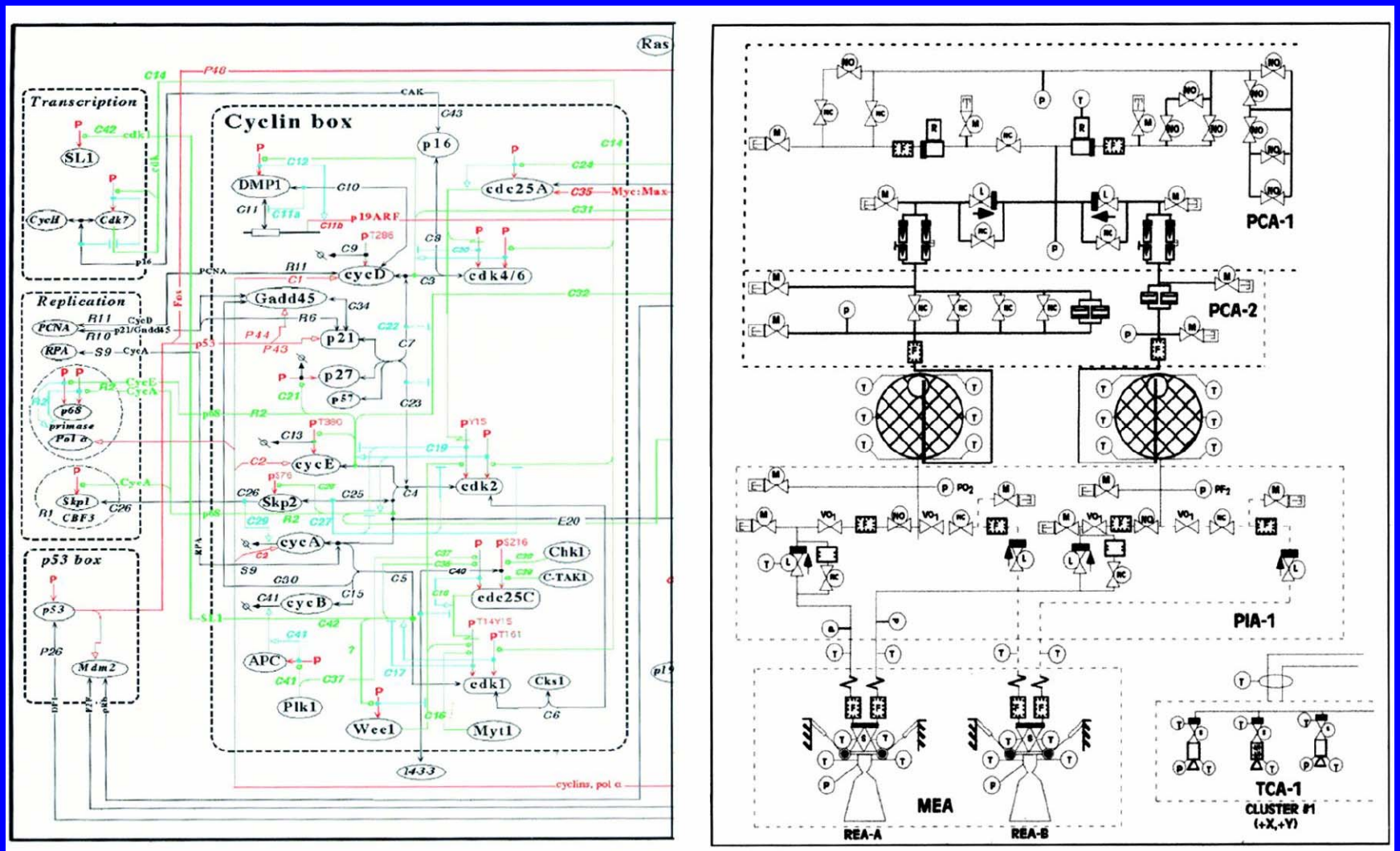
The Cell Therapy Bench to Bedside Process



P. Smith et al.
NIDDK

Cell Science

Rocket Science



Roger Brent: Genomic Biology CELL 100:169, 2000